# MeiraGTx Announces $50 Million Offering of Ordinary Shares led by Sanofi and Reports Second Quarter 2024 Financial and Operational Results

August 12, 2024

*- Positive data from the Phase 1 AQUAx study in radiation-induced xerostomia (RIX) presented at the American Academy of Oral Medicine 2024 annual meeting (AAOM) showed meaningful improvements in patient-reported outcomes and saliva production with AAV2-hAQP1 treatment*

*- Company awarded Innovation Passport Designation by the U.K. Innovative Licensing and Access Pathway Steering Group for AAV8-RK-AIPL1 for the treatment of AIPL1-Leber congenital amaurosis 4 (LCA4)*

LONDON and NEW YORK, Aug. 12, 2024 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical-stage genetic medicine company, today announced financial and operational results for the second quarter ended June 30, 2024, and provided a corporate update. The Company also announced that it has agreed to sell 12.5 million ordinary shares at a price of $4.00 per share. MeiraGTx anticipates aggregate gross proceeds from the offering will be $50 million.

The financing was led by Sanofi, which made a $30 million equity investment in the Company through the offering. Other participants included Perceptive Advisors and leading institutional healthcare funds. The offering is expected to close on or about August 13, 2024, subject to customary closing conditions.

“We are very pleased to receive additional investment from Sanofi and other investors,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “The additional funds will allow us to accelerate development of our riboswitch *in vivo* delivery platform to the clinic with our completely novel and differentiated approach to treating obesity and metabolic disease.”

Dr. Forbes continued, “Our lead clinical programs are all progressing well with several important milestones coming before the end of this year. We continue to enroll our pivotal Phase 2 AQUAx2 clinical trial for Grade 2/3 radiation-induced xerostomia that could support a potential BLA filing in 2026. We also anticipate results from our blinded, placebo controlled bridging study of AAV-GAD for Parkinson’s disease which will allow discussions with global regulatory agencies on the Phase 3 clinical program.”

Dr. Forbes continued, “We anticipate data from the Phase 3 LUMEOS trial of bota-vec for XLRP in collaboration with Johnson & Johnson Innovative Medicine (formally known as Janssen) this year. We are eligible to receive up to $285 million upon the first commercial sales of bota-vec in the U.S. and EU and manufacturing tech transfer. Additionally, our riboswitch *in vivo* delivery platform continues to show encouraging data in obesity and metabolic disease as well as CAR-T and other areas, and we look forward to sharing updates later this year.”

Dr. Forbes concluded, “Finally, we are very excited to have been awarded an Innovation Passport Designation for AAV8-RK-AIPL1 for the treatment of LCA4, granting us entry into the U.K.’s Innovative Licensing and Access Pathway (ILAP). We are working closely with the ILAP Steering Group to advance AAV8-RK-AIPL1 as quickly as possible towards potential approval and ultimately deliver it to babies who were previously deemed untreatable and destined to be blind for life. The results from the 11 infants and toddlers treated to date are truly remarkable, with every one of the children treated who were all blind at birth now having visual acuity.”

**Recent Development Highlights and Anticipated Milestones**

**AAV2-hAQP1 for the Treatment of Xerostomia:**

- Data from the Company’s Phase 1 AQUAx clinical trial were presented in an oral session at the AAOM 2024 annual meeting in April, demonstrating that treatment with AAV2-hAQP1 resulted in significant improvements across three different patient-reported outcomes and in saliva production, with no treatment-related serious adverse events or dose-limiting toxicities reported.
- The Company continues to enroll and dose participants at multiple sites in the U.S., Canada and the U.K. in the Phase 2 AQUAx2 (NCT05926765) randomized, double-blind, placebo-controlled study.
- The Company recently gained alignment with the FDA on requirements for the ongoing Phase 2 AQUAx2 clinical trial for Grade 2/3 radiation-induced xerostomia to be considered a pivotal trial in support of a potential BLA filing.

**AAV-GAD for the Treatment of Parkinson’s Disease:**

- The Company completed dosing patients in the Phase 1 trial of AAV-GAD under a new IND with material manufactured in its GMP facility in London, U.K. using MeiraGTx’s proprietary production process in Q1 2024.
- The Company anticipates results from the study in the fourth quarter of 2024. The AAV-GAD trial is a three-arm randomized clinical bridging study with subjects randomized to sham control or one of two doses of AAV-GAD to evaluate the safety and tolerability of AAV-GAD when delivered to the subthalamic nucleus (STN) of patients with Parkinson’s disease (NCT05603312).

- The Company intends to initiate discussions with global regulatory agencies in the fourth quarter 2024 around the Phase 3 clinical program.

**Bota-vec for the Treatment of XLRP:**

- Data from the Phase 1/2 study of bota-vec in XLRP was published in the American Journal of Ophthalmology. The study showed that treatment with bota-vec led to improvements in functional vision, as well as retinal and visual functions, compared to untreated controls. The article "*Phase 1/2 AAV5-hRKp.RPGR (Botaretigene Sparoparvovec) Gene Therapy: Safety and Efficacy in RPGR-Associated X-Linked Retinitis Pigmentosa*" is available online.

- MeiraGTx anticipates receiving an additional $15 million in milestone payments later in 2024 and will receive up to a further $285 million upon first commercial sales of bota-vec in the U.S. and EU and for manufacturing technology transfer.

- MeiraGTx also entered into a commercial supply agreement with Johnson & Johnson Innovative Medicine for bota-vec manufacturing, which the Company anticipates will generate additional revenue during the product launch.

**AAV-AIPL1 Specials License in the U.K.:**

- MeiraGTx was awarded an Innovation Passport Designation by the U.K. Innovative Licensing and Access Pathway Steering Group for AAV8-RK-AIPL1.
    - Designation provides entry into the U.K.'s Innovative Licensing and Access Pathway (ILAP) designed to accelerate time to market and patient access to innovative medicines.
    - Other benefits of ILAP include access to a range of development tools, such as the potential for accelerated Marketing Authorization Application (MAA) assessment, rolling review, and a continuous benefit-risk assessment, or potential Marketing Authorization under Exceptional Circumstances.

- Meaningful responses have been observed in 11 out of 11 LCA4 children treated to date with AAV-AIPL1. All children were treated between 1 and 3 years old, all were blind on treatment, and all gained visual acuity 4 or more weeks following treatment.

- The Company's AAV-AIPL1 for the treatment of inherited retinal dystrophy due to defects in the *AIPL1* gene has been granted orphan drug designation by the FDA and orphan designation by the European Commission.

**Riboswitch Gene Regulation Technology: Upcoming R&D Day**

- Later this year, the Company intends to present data from its riboswitch gene regulation technology platform for *in vivo* delivery at an R&D Day highlighting encouraging data in metabolic disease models as well as CAR-T for both oncology and autoimmune diseases:

    - ***Obesity and metabolic disease***: The company has successfully delivered multiple combinations of gut peptides *in vivo* including GLP-1, GIP, PYY, Glucagon, and Oxyntomodulin as well as novel myokine and adipokine peptides that drive muscle metabolism and fat storage, via the riboswitch platform. This proprietary *in vivo* delivery technology allows daily dosing with a small molecule to drive the production of natural short lived peptides within the body in physiologically relevant combinations and timing. This provides a platform for addressing not just weight loss via reduced appetite, but also muscle strength, fat metabolism, cardiovascular health and neurodegenerative disorders in metabolic disease, with daily oral small molecules.
    - ***CAR-T for both oncology and autoimmune disease***: Precise control of levels and timing of the CAR with our riboswitch platform has demonstrated a significant impact on CAR-T efficacy, with a 3-4 fold improvement in *in vivo* potency of T-cells with regulated CAR compared to the currently approved CAR-T with unregulated constitutively active CAR. In addition, MeiraGTx's regulated CAR-T displays a normal naïve T-cell profile, lacking exhaustion markers and retaining proliferation and killing ability in contrast to CAR-T with unregulated constitutive CAR expression.

As of June 30, 2024, MeiraGTx had cash and cash equivalents of approximately $100.0 million as well as approximately $1.6 million in receivables due from Johnson & Johnson Innovative Medicine. The Company believes that with such funds, as well as anticipated near-term milestones from Johnson & Johnson Innovative Medicine under the asset purchase agreement, together with the proceeds from the offering and tax incentive receivable, it will have sufficient capital to fund operating expenses and capital expenditure requirements into the second quarter of 2026. This estimate does not include the $285.0 million in milestones the Company is eligible to receive under the asset purchase agreement upon first commercial sale of bota-vec in the United States and in at least one of the United Kingdom, France, Germany, Spain and Italy, and for completion of the transfer of certain manufacturing technology.

**Financial Results**

Cash, cash equivalents and restricted cash were $101.0 million as of June 30, 2024, compared to $130.6 million as of December 31, 2023.

Service revenue was $0.3 million for the three months ended June 30, 2024 due to progress of process performance qualification services under the

asset purchase agreement with Johnson & Johnson Innovative Medicine.

There was no license revenue for the three months ended June 30, 2024, compared to $3.5 million for the three months ended June 30, 2023. The decrease is due to the termination of the collaboration agreement concurrent with the execution of the asset purchase agreement with Johnson & Johnson Innovative Medicine.

General and administrative expenses were $11.3 million for the three months ended June 30, 2024, compared to $12.4 million for the three months ended June 30, 2023. The decrease of $1.1 million was primarily due to a decrease in share-based compensation, payroll and payroll-related costs, insurance costs and rent and facilities costs. These decreases were partially offset by an increase in consulting fees and other office related costs.

Research and development expenses for the three months ended June 30, 2024 were $34.9 million, compared to $19.9 million for the three months ended June 30, 2023. The increase of $15.0 million was primarily due to a decrease in reimbursements from Johnson & Johnson Innovative Medicine as the reimbursement for the three months ended June 30, 2023 was in connection with research funding provided under the collaboration agreement, which was terminated on December 20, 2023, whereas the reimbursement for the three months ended June 30, 2024 was in connection with transition services we provided to Johnson & Johnson Innovative Medicine. Additionally, expenses related to our preclinical programs increased primarily related to development of our gene regulation technology. These increases were partially offset by decreases in manufacturing costs primarily due to an increase in the number of batches of clinical trial material produced during the three months ended June 30, 2024 compared to the three months ended June 30, 2023, which costs were charged to the clinical programs, a decrease in other research and development costs, as well as a decrease in clinical trial expenses primarily related to bota-vec as Johnson & Johnson Innovative Medicine is now primarily funding the expenses related to this program as a result of the asset purchase agreement. The decrease in expenses related to bota-vec were partially offset by an increase in expenses related to our other clinical programs, primarily AAV-hAQP1.

Foreign currency loss was $0.3 million for the three months ended June 30, 2024, compared to a gain of $1.9 million for the three months ended June 30, 2023. The change of $2.2 million was primarily due to the restructuring and payment of certain intercompany receivables and payables. Foreign currency gains and losses subsequent to the restructuring are recorded as a part of accumulated other comprehensive income.

Interest income was $0.8 million for the three months ended June 30, 2024, compared to $0.7 million for the three months ended June 30, 2023. The increase of $0.1 million was due to higher interest rates and cash balances during 2024.

Interest expense was $3.3 million for each of the three months ended June 30, 2024 and June 30, 2023.

Net loss attributable to ordinary shareholders for the quarter ended June 30, 2024, was $48.6 million, or $0.76 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $29.6 million, or $0.53 basic and diluted net loss per ordinary share for the quarter ended June 30, 2023.

**About MeiraGTx**
MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage genetic medicine company with a broad pipeline of late-stage clinical programs supported by end-to-end manufacturing capabilities. MeiraGTx has an internally developed manufacturing platform process, internal plasmid production for GMP, two GMP viral vector production facilities as well as an in-house Quality Control hub for stability and release, all fit for IND through commercial supply. MeiraGTx has core capabilities in viral vector design and optimization and a potentially transformative riboswitch gene regulation platform technology that allows for the precise, dose-responsive control of gene expression by oral small molecules. MeiraGTx is focusing the riboswitch platform on delivery of metabolic peptides including GLP-1, GIP, Glucagon and PYY using oral small molecules, as well as cell therapy for oncology and autoimmune diseases. Although initially focusing on the eye, central nervous system, and salivary gland, MeiraGTx has developed the technology to apply genetic medicine to more common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains great.

For more information, please visit www.meiragtx.com.

**Forward Looking Statement**
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding our product candidate development, our ability to manufacture product candidates, potential milestone payments and the achievement of such milestones, including the receipt of such milestone payments and the impact on our cash runway, and our pre-clinical and clinical data, reporting of such data and the timing of results of data and regulatory matters, as well as statements that include the words “expect,” “will,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “could,” “should,” “would,” “continue,” “anticipate” and similar statements of a future or forward-looking nature. These forward-looking statements are based on management’s current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, our incurrence of significant losses; any inability to achieve or maintain profitability, raise additional capital, repay our debt obligations, identify additional and develop existing product candidates, successfully execute strategic transactions or priorities, bring product candidates to market, expansion of our manufacturing facilities and processes, successfully enroll patients in and complete clinical trials, accurately predict growth assumptions, recognize benefits of any orphan drug designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of pandemics, epidemics or outbreaks of infectious diseases on the status, enrollment, timing and results of our clinical trials and on our business, results of operations and financial condition; failure of early data to predict eventual outcomes; failure to obtain FDA or other regulatory approval for product candidates within expected time frames or at all; the novel nature and impact of negative public opinion of gene therapy; failure to comply with ongoing regulatory obligations; contamination or shortage of raw materials or other manufacturing issues; changes in healthcare laws; risks associated with our international operations; significant competition in the pharmaceutical and biotechnology industries; dependence on third parties; risks related to intellectual property; changes in tax policy or treatment; our ability to utilize our loss and tax credit carryforwards; litigation risks; and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2024, as such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC’s website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, unless required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Thus, one should not assume that our silence over time means that

actual events are bearing out as expressed or implied in such forward-looking statements. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

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**MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES**
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**
**(unaudited)**
**(in thousands, except share and per share amounts)**

| | For the Three-Month Periods Ended June 30, | | For the Six-Month Periods Ended June 30, | |
|---|---|---|---|---|
| | 2024 | 2023 | 2024 | 2023 |
| Revenues: | | | | |
| Service revenue - related party | $ 282 | $ — | $ 979 | $ — |
| License revenue - related party | — | 3,540 | — | 6,874 |
| Total revenue | 282 | 3,540 | 979 | 6,874 |
| Operating expenses: | | | | |
| General and administrative | 11,257 | 12,388 | 24,404 | 25,160 |
| Research and development | 34,934 | 19,937 | 69,256 | 42,259 |
| Total operating expenses | 46,191 | 32,325 | 93,660 | 67,419 |
| Loss from operations | (45,909) | (28,785) | (92,681) | (60,545) |
| Other non-operating income (expense): | | | | |
| Foreign currency (loss) gain | (284) | 1,905 | (819) | 5,762 |
| Interest income | 827 | 655 | 1,924 | 1,200 |
| Interest expense | (3,254) | (3,355) | (6,504) | (6,415) |
| Gain on sale of nonfinancial assets | — | — | 29,018 | — |
| Fair value adjustment | — | (1) | — | 53 |
| Net loss | (48,620) | (29,581) | (69,062) | (59,945) |
| Other comprehensive loss: | | | | |
| Foreign currency translation loss | (488) | (2,541) | (2,179) | (4,894) |
| Comprehensive loss | $ (49,108) | $ (32,122) | $ (71,241) | $ (64,839) |
| | | | | |
| Net loss | $ (48,620) | $ (29,581) | $ (69,062) | $ (59,945) |
| Basic and diluted net loss per ordinary share | $ (0.76) | $ (0.53) | $ (1.08) | $ (1.15) |
| Weighted-average number of ordinary shares outstanding | 64,376,396 | 55,349,534 | 64,221,145 | 52,012,382 |

**MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES**
**CONDENSED CONSOLIDATED BALANCE SHEETS**
**(unaudited)**
**(in thousands, except share and per share amounts)**

| | June 30, 2024 | December 31, 2023 |
|---|---|---|
| ASSETS | | |
| CURRENT ASSETS: | | |

| | | |
|---|---|---|
| Cash and cash equivalents | $ 99,974 | $ 129,566 |
| Accounts receivable - related party | 1,628 | 10,138 |
| Prepaid expenses | 4,955 | 5,625 |
| Tax incentive receivable | 3,557 | 13,277 |
| Other current assets | 660 | 1,016 |
| Total Current Assets | 110,774 | 159,622 |
| | | |
| Property, plant and equipment, net | 108,844 | 115,896 |
| Intangible assets, net | 969 | 1,118 |
| Restricted cash | 1,051 | 1,083 |
| Other assets | 1,139 | 1,917 |
| Equity method and other investments | 6,766 | 6,766 |
| Right-of-use assets - operating leases, net | 13,823 | 15,910 |
| Right-of-use assets - finance leases, net | 23,285 | 24,432 |
| TOTAL ASSETS | $ 266,651 | $ 326,744 |
| | | |
| LIABILITIES AND SHAREHOLDERS' EQUITY | | |
| CURRENT LIABILITIES: | | |
| Accounts payable | $ 21,398 | $ 16,042 |
| Accrued expenses | 16,928 | 42,639 |
| Lease obligations, current | 4,212 | 4,193 |
| Deferred revenue - related party, current | 3,498 | 2,926 |
| Other current liabilities | 970 | 1,278 |
| Total Current Liabilities | 47,006 | 67,078 |
| Deferred revenue - related party | 53,763 | 34,017 |
| Lease obligations | 10,688 | 12,952 |
| Asset retirement obligations | 2,490 | 2,401 |
| Note payable, net | 72,665 | 72,119 |
| TOTAL LIABILITIES | 186,612 | 188,567 |
| COMMITMENTS AND CONTINGENCIES (Note 11) | | |
| | | |
| SHAREHOLDERS' EQUITY: | | |
| Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 64,684,187 and 63,601,015 shares issued and outstanding at June 30, 2024 and December 31, 2023, respectively | 3 | 2 |
| Capital in excess of par value | 706,943 | 693,841 |
| Accumulated other comprehensive loss | (3,614) | (1,435) |
| Accumulated deficit | (623,293) | (554,231) |
| Total Shareholders' Equity | 80,039 | 138,177 |
| TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY | $ 266,651 | $ 326,744 |